CLAIMS

1. An isolated nucleic acid molecule comprising first and second domains, said first domain being capable of forming a first specific binding pair with a target sequence of a target human RNA species within 100 nucleotides of an RNA processing or translation site on the RNA target species, said second domain consisting of a sequence which forms a second specific binding pair with at least one RNA processing or translation factor, wherein formation of the first specific binding pair and the second specific binding pair recruits the RNA processing or translation factor to the RNA processing or translation site on the human RNA target species to effect RNA processing or translation at said RNA processing or translation site.

2. A nucleic acid molecule according to claim 1 wherein the RNA processing or translation site on the RNA target species is selected from an RNA splicing site, a cryptic RNA splicing site, a polyadenylation site and a translation initiation site.

3. A nucleic acid molecule according to claim 1 or 2 wherein the RNA processing or translation site on the RNA target species is mutated.

4. A nucleic acid molecule according to any of claims 1 to 3 wherein a further site on the target RNA species is mutated, wherein the further site contributes to a protein or RNA-protein assembly required for processing or translation at the RNA processing or translation site.

5. A nucleic acid molecule according to any of claims 1 to 4 wherein said first domain of said nucleic acid molecule attaches to said target sequence of said RNA target species by means of complementary base pairing.

6. A nucleic acid molecule according to any of claims 1 to 5 wherein said second domain forms a second specific binding pair with an RNA processing or translation factor selected from the group consisting of: RNA molecules, RNA structural molecules, RNA stability molecules, splicing factors, polyadenylation factors, transcription factors, and translation factors, and combinations thereof.

7. A nucleic acid molecule according to any of claims 1 to 6 wherein said second domain forms a second specific binding pair with an RNA processing factor which is any RNA or protein that stimulates splicing activity.

8. A nucleic acid molecule according to any of claims 1 to 7, wherein said second domain forms a second specific binding pair with an RNA processing factor selected from the group consisting of: SR proteins, SR-related proteins and hnRNP proteins.

9. A nucleic acid molecule according to any of claims 1 to 8, wherein said second domain forms a second specific binding pair with an RNA processing factor selected from the UsnRNP group of RNA splicing factors.

10. A nucleic acid molecule according to claim 9, wherein said second domain forms a second specific binding pair with U1 or U2 snRNP.

11. A nucleic acid molecule according to any of claims 1 to 5, wherein said second domain forms a second specific binding pair with an RNA translation factor selected an initiation factor, such as eIF4G and eIF3, or a ribosomal component.

12. A nucleic acid molecule according to any of the preceding claims wherein said second domain is not complementary to the target RNA species.

13. A nucleic acid molecule according to any of claims 1 to 12, wherein said nucleic acid molecule comprises at least one modified nucleotide.

14. A nucleic acid molecule according to claim 13, wherein said at least one modified nucleotide is chemically modified to enhance stability or uptake by a cell.

15. A nucleic acid molecule according to claim 13 or 14, wherein said at least one modified nucleotide is selected from the group consisting of a 2'-*O*-methyl

derivative of RNA, a phosphothiorate modification, a morpholino modification and a phosphoroamidate modification.

16. A polynucleotide that encodes the nucleic acid molecule according to any of claims 1 to 12.

17. A vector that comprises the polynucleotide of claim 16.

18. A host cell or stable cell line that comprises the vector of claim 17.

19. A pharmaceutical composition comprising the nucleic acid molecule according to any of claims 1 to 15, or the polynucleotide of claim 16, or the vector of claim 17, and a pharmaceutially acceptable carrier, diluent or excipient, or in a pharmaceutically acceptable delivery system.

20. The nucleic acid molecule according to any of claims 1 to 15, or the polynucleotide of claim 16, or the vector of claim 17, for use in medicine.

21. A method of recruiting an RNA processing or translation factor to a target RNA species, the method comprising:

providing a nucleic acid molecule having a first domain capable of forming a first specific binding pair with a target sequence on the target RNA species, and a second domain capable of forming a second specific binding pair with an RNA processing or translation factor, and

contacting the nucleic acid molecule with the target RNA species and with the RNA processing or translation factor.

22. Use of a nucleic acid molecule having a first domain capable of forming a first specific binding pair with a target sequence on the target RNA species, and a second domain capable of forming a second specific binding pair with an RNA processing or translation factor, in the preparation of a medicament for recruiting an RNA processing or translation factor to the target RNA species.

23. Use of a nucleic acid molecule having a first domain capable of forming a first specific binding pair with a target sequence on the target RNA species, and a second domain capable of forming a second specific binding pair with an RNA processing or translation factor, for recruiting an RNA processing or translation factor to the target RNA species.

24. A method or a use according to any of claims 21 to 23 wherein formation of the first specific binding pair and the second specific binding pair recruits the RNA processing or translation factor to an RNA processing or translation site on the target RNA species to effect RNA processing or translation at said RNA processing or translation site.

25. A method or a use according to any of claims 21 to 24 wherein the target sequence is within 100 nucleotides of an RNA processing or translation site on the RNA target species.

26. A method or a use according to any of claims 21 to 25 wherein the RNA processing or translation factor is selected from the group consisting of: RNA molecules, RNA structural molecules, RNA stability molecules, splicing factors, polyadenylation factors, transcription factors, and translation factors, and combinations thereof.

27. A method or a use according to any of claims 21 to 26 for increasing the level of splicing at a specific splice site on a target RNA species, wherein the first domain of the nucleic acid molecule forms a specific binding pair with a target sequence close to the specific splice site on the RNA species, and wherein the second domain forms a specific binding pair with an RNA splicing factor.

28. A method or a use according to claim 27 wherein the specific splice site is a cryptic splice site or a mutated splice site.

29. A method according to any of claims 21 to 26 for increasing the level of incorporation of a specific exon in a pre-mRNA species into a mature mRNA

species, wherein the first domain of the nucleic acid molecule forms a specific binding pair with a target sequence in the specific exon of the pre-mRNA species, and wherein the second domain forms a specific binding pair with an RNA splicing factor.

30. A method or a use according to any of claims 27 to 29 wherein the RNA splicing factor is selected from the group consisting of: SR proteins, SR-related proteins and hnRNP proteins, and any RNA or protein that stimulates splicing activity.

31. A method according to any of Claims 21 or 24 to 30, or a use according to claim 23, which is performed in an *in vitro* cell-free system.

32. A method according to any of Claims 21 or 24 to 30, or a use according to claim 23, which is performed in an *ex vivo* cellular system.

33. A method according to any of Claims 21 or 24 to 30 which is performed *in vivo* in the human or animal body.

34. A method of treating a condition characterised by defective or undesirable RNA splicing in an individual, the method comprising administering to the individual a nucleic acid molecule having a first domain capable of forming a specific binding pair with a target region of a defectively spliced target RNA species and having a second domain that forms a specific binding pair with an RNA splicing factor, wherein the target region of the target RNA species is sufficiently close on the RNA species to the site of defective or undesirable splicing for splicing at the site to be enhanced by the action of the splicing factor.

35. Use of a nucleic acid molecule having a first domain capable of forming a specific binding pair with a target region of a defectively spliced target RNA species and having a second domain that forms a specific binding pair with an RNA splicing factor, in the preparation of a medicament for treating a condition characterised by defective or undesirable RNA splicing of the target RNA species, wherein said target

region is sufficiently close on the RNA species to the site of defective or undesirable splicing for splicing at the site to be enhanced by the action of the splicing factor.

36. A method or a use according to claim 34 or 35 wherein the RNA splicing factor is selected from the group consisting of: SR proteins, SR-related proteins, and hnRNP proteins, and any RNA or protein that stimulates splicing activity.

37. A method or a use according to any of claims 34 to 36 wherein the defective RNA splicing is caused by a mutation at the site of defective splicing.

38. A method or a use according to any of claims 34 to 36 wherein enhanced exonic incorporation is desirable at the site of undesirable RNA splicing.

39. A method or a use according to any of claims 34 to 38 wherein the condition is selected from spinal muscular atrophy, Becker muscular dystrophy and β-thalassaemia.

40. A method of treating a condition characterised by inadequate or defective translation of an RNA species in an individual, the method comprising administering to the individual a nucleic acid molecule having a first domain capable of forming a specific binding pair with a target region of an inadequately or defective translated target RNA species and having a second domain that forms a specific binding pair with an RNA translation factor, wherein said target region of the target RNA species is sufficiently close on the RNA species to a translation initiation site for translation at the site to be enhanced by the action of the translation factor.

41. Use of a nucleic acid molecule having a first domain complementary to a target region of an inadequately or defective translated target RNA species and having a second domain that forms a specific binding pair with an RNA translation factor, in the preparation of a medicament for treating a condition characterised by inadequate or defective translation of an RNA species, wherein said target region is sufficiently close on the RNA species to a translation initiation site for translation at the site to be enhanced by the action of the translation factor.

42. A method or a use according to Claim 40 or 41 wherein the RNA translation factor is selected from the group consisting of an initiation factor such as eIF4G and eIF3 or a ribosomal component.

43. A method of enhancing polyadenylation at a desired polyadenylation site on a target RNA species, the method comprising:

providing a nucleic acid molecule having a first domain that is capable of forming a first specific binding pair with a target sequence close to the desired polyadenylation site on the target RNA species, and a second domain that is capable of forming a first specific binding pair with an RNA polyadenylation factor, and

contacting the nucleic acid molecule with the target RNA species and with the RNA splicing factor.

44. Use of a nucleic acid molecule having a first domain that is capable of forming a first specific binding pair with a target sequence close to the desired polyadenylation site on a target RNA species, and a second domain that is capable of forming a first specific binding pair with an RNA polyadenylation factor, in the preparation of a medicament for increasing the level of polyadenylation at a desired polyadenylation site on the target RNA species.

45. A method or a use according to Claim 43 or 44 wherein the RNA polyadenylation factor is cleavage and polyadenylation specificity factor (CPSF).

46. A method or a use according to any of Claims 21 to 45 wherein the nucleic acid molecule is as defined in any of Claims 1 to 15.

47. The use of a nucleic acid molecule according to any of claims 1 to 15 in the manufacture of a medicament for the treatment of RNA processing or translation defects of the human or animal body caused by mutations in RNA that affect binding of RNA processing or translation factors.

48. A method for the manufacture of a medicament for the treatment of RNA processing or translation defects caused by mutations in RNA that affect binding of RNA processing or translation factors, characterised in the use of a nucleic acid molecule according to any of claims 1 to 15.

49. A method for the treatment of RNA processing or translation defects caused by mutations in RNA that affect binding of RNA processing or translation factors comprising administering to a patient a medicament made according to the method of claim 48.

54. A method of affecting RNA processing or translation in an *in vitro* system characterised in the use of a nucleic acid molecule according to any of claims 1 to 15.

51. A method of designing a nucleic acid molecule that affects RNA processing or translation at an RNA processing or translation site on a target RNA species, the method comprising:

(a) identifying the RNA processing or translation site on the target RNA species, and

(b) designing an oligonucleotide molecule comprising:

(i) a nucleotide sequence that forms a specific binding pair with a target sequence close to the RNA processing or translation site on the target RNA species, and

(ii) a nucleotide sequence motif that forms a specific binding pair with an RNA processing or translation factor which affects processing or translation of the target RNA species at the RNA processing or translation site.

52. A method according to claim 51 further comprising the prior step of selecting a target RNA species.

53. A method of making a nucleic acid molecule that affects RNA processing or translation at an RNA processing or translation site on a target RNA species, the

method comprising designing a nucleic acid molecule according to Claim 51 or 52 and synthesizing the nucleic acid molecule.

54. A method of making a nucleic acid molecule that affects RNA processing or translation at an RNA processing or translation site on a target RNA species, the method comprising designing a nucleic acid molecule according to Claim 51 or 52 and expressing the nucleic acid molecule from a polynucleotide encoding it.

55. A method according to any of claims 51 to 54 wherein the target RNA species is transcribed from a defective or mutated disease gene.

56. A nucleic acid molecule obtainable by the method of any of claims 53 to 55.